EXHIBIT F

Trial Review

< BACK

Trial from ClinicalTrials.gov

For full trial details, please see the original record at https://clinicaltrials.gov/show/NCT01494805

Trial ID	NCT01494805
Ethics application status	
Date submitted	14/12/2011
Date registered	14/12/2011

Titles & IDs

Public title	A Phase I/II Controlled Dose-escalating Trial to Establish the Baseline Safety and Efficacy of a Single Subretinal Injection of rAAV.sFlt-1 Into Eyes of Patients With Exudative Age-related Macular Degeneration (AMD)
Scientific title	Safety and Efficacy Study of rAAV.sFlt-1 in Patients With Exudative Age-Related Macular Degeneration

EXHIBIT F

Secondary ID [1]	2008-135
Universal Trial Number (UTN)	
Trial acronym	AMD
Linked study record	

Health condition

Health condition(s) or problem(s) studied:

Condition category	Condition code
Macular Degeneration	
Age-related Maculopathies	
Age-related Maculopathy	
Maculopathies,Age-related	
Maculopathy,Age-related	
Retinal Degeneration	
Retinal Neovascularization	
Eye Diseases	

Intervention/exposure

Study type	Interventional
Patient registry	
Target follow-up duration	
Target follow-up type	
Description of intervention(s) / exposure	A new treatment for exudative age-related macular degeneration (wet AMD) is being investigated. The purpose of this Phase I/II clinical research study is to examine the baseline safety and efficacy of an experimental study drug to treat a complication of the disease which leads to vision loss. The name of the study drug is rAAV.sFlt-1. This experimental study uses a non-pathogenic virus to express a therapeutic protein within the eye. The therapeutic diminishes the growth of abnormal blood vessels under the retina. The duration of effect is thought to be long-term (years) following a single administration.

EXHIBIT F

The clinical research study will look at the baseline safety and efficacy of a single injection of rAAV.sFlt-1 injected directly into the eye.

Approximately forty (40) subjects will participate in Australia. The primary endpoint of the study is at one month, with extended follow up for 3 years.
Allocation: Randomized, Endpoint Classification: Safety/Efficacy Study, Intervention Model: Parallel Assignment, Masking: Single Blind (Outcomes Assessor), Primary Purpose: Treatment

Intervention code [1] Biological - rAAV.sFlt-1

Intervention code [2] Biological - rAAV.sFlt-1

Intervention code [3] Other - Control (ranibizumab alone)

Comparator / control treatment

Control group

Outcomes

Primary outcome [1] No sign of unresolved ophthalmic complications, toxicity or systemic complications as measured by laboratory tests from 1 month post injection

Timepoint [1] Primary endpoint at 1 month

Secondary outcome [1] Maintenance or improvement of vision without the necessity of ranibizumab re-injections

Timepoint [1] Up to 3 years

Eligibility

Key inclusion criteria

Inclusion Criteria:

- Age greater than or equal to 55 years;

- Subfoveal CNV secondary to AMD and with best corrected visual acuity of 3/60 - 6/9 with 6/60 or better in the other eye;

- Fluorescein angiogram of the study eye must show evidence of a leaking subfoveal choroidal neovascular lesion, or CNV currently under active management with anti-VEGF therapy;

EXHIBIT F

- Must be a candidate for anti-VEGF intravitreal injections;

- No previous retinal treatment of photodynamic therapy or laser;

- Able to provide informed consent;

- Able to comply with protocol requirements, including follow-up visits.

Minimum age	55 Years
Maximum age	N/A
Gender	Both
Can healthy volunteers participate?	No
Key exclusion criteria	Exclusion Criteria:

- Liver enzymes > 2 X upper limit of normal;

- Any prior treatment for AMD in the study / control eye, excluding anti-VEGF injections;

- Extensive sub-foveal scarring, extensive geographic atrophy, or thick subretinal blood in the study eye as determined by the investigator;

- Significant retinal disease other than sub-foveal CNV AMD;

Study design

Purpose

Duration

Selection

Timing

Statistical methods / analysis

Recruitment

EXHIBIT F

Recruitment status	Active, not recruiting
Data analysis	
Reason for early stopping/withdrawal	
Other reasons	
Anticipated date of first participant enrolment	
Actual date of first participant enrolment	31/12/2011
Anticipated date last participant enrolled	
Actual date last participant enrolled	
Anticipated date of last data collection	
Actual date of last data collection	
Target sample size	40
Actual sample size	

Recruitment in Australia

Recruitment state(s)	WA
Recruitment hospital [1]	Lions Eye Institute - Nedlands - 6009

Funding & Sponsors

Primary sponsor type	Other
Name	Lions Eye Institute, Perth, Western Australia
Address	
Country	
Secondary sponsor category [1]	Industry
Name [1]	Avalanche Biotechnologies, Inc.

EXHIBIT F

Address [1]

Country [1]

Ethics approval

Ethics application status

Summary

Brief summary	The study will involve approximately 40 subjects aged 55 or above who have exudative age-related macular degeneration (wet AMD). Patients will be randomized to receive one of two doses of rAAV.sFlt-1 or assigned to the control group.
Trial website	https://clinicaltrials.gov/show/NCT01494805
Trial related presentations / publications	
Public notes	

Contacts

Principal investigator

Contact person for public queries

Contact person for scientific queries

< BACK

EXHIBIT F